MHRA
10 South Colonnade
Canary Wharf
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-101467-PIP01-24

Scope of the Application

Active Substance(s)

apitegromab

Condition(s)

Treatment of spinal muscular atrophy

Pharmaceutical Form(s)

Concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Scholar Rock, Inc.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Scholar Rock, Inc. submitted to the licensing authority on 08/07/2024 16:53 BST an application for a Paediatric Investigation Plan

The procedure started on 03/09/2024 08:33 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral and grant a waiver

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-101467-PIP01-24

Of 25/10/2024 08:27 BST

On the adopted decision for apitegromab (MHRA-101467-PIP01-24) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for apitegromab, Concentrate for solution for infusion , INTRAVENOUS USE .

This decision is addressed to Scholar Rock, Inc., 301 Binney St 3rd Floor, Cambridge, MA, UNITED STATES OF AMERICA, 02142

ANNEX I

1. Waiver

1.1 Condition:

Treatment of spinal muscular atrophy The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 months of age Pharmaceutical form(s): Concentrate for solution for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of spinal muscular atrophy

2.2 Indication(s) targeted by the PIP:

Treatment of spinal muscular atrophy

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 2 months to less than 18 years of age

2.4 Pharmaceutical Form(s):

Concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	1	Study 1 Prenatal and Postnatal Development study including maternal function with apitegromab in rats.
Clinical Studies	3	Study 2 (SRK-015-002 [TOPAZ]) Open-label, uncontrolled trial to evaluate safety and activity of apitegromab in children from 2 years to less than 18 years of age (and adults) with spinal muscular atrophy (type 2 and type 3 SMA). Study 3 (SRK-015-003 [SAPPHIRE]) Double-blind, randomised, placebo-controlled trial to evaluate safety, tolerability and efficacy of apitegromab in children from 2 years to less than 18 years of age with spinal muscular atrophy (type 2 and type 3 SMA). Study 4 (SRK-015-005) Double-blind, randomised trial to evaluate pharmacokinetics, safety and efficacy of 2 different doses of apitegromab in children from 2 months to less than 2 years of age with spinal muscular atrophy (type 1 and pre-symptomatic SMA).
Extrapolation, Modeling & Simulation Studies	2	Study 5 Modelling and simulation study to support dose finding of apitegromab in children from 2 months to less than 2 years of age with spinal muscular atrophy

		(type 1 SMA). Study 6 Modelling and simulation study to support final dosing recommendations for apitegromab in the treatment of SMA in children from 2 months to less than 2 years of age.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	31/12/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes